

FDA Drug Topics: Project Facilitate: An Oncology Expanded Access Program Update



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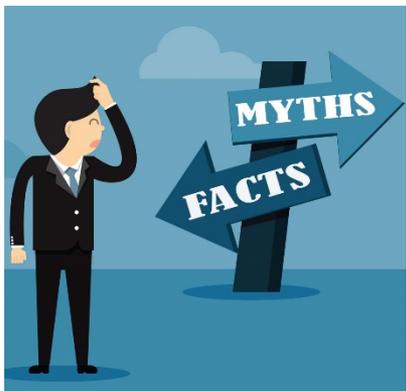


Disclosure Information

Mitchell Chan: Nothing to disclose

Objectives

1. Define expanded access and the key requirements for an expanded access request for an individual patient.
2. Summarize the key responsibilities of the oncology healthcare professional when considering expanded access for a patient.
3. Identify resources available to healthcare professionals considering submission of an oncology expanded access request for an individual patient.
4. Describe how Project Facilitate is a resource to navigate the oncology Expanded Access pathway



Misconceptions

Myth: Expanded Access (EA) is inaccessible because the oncologist I work with is too busy to do the paperwork and we don't have a regulatory office

Fact: *Project Facilitate (PF) works with any regulatory or healthcare professional through the process, help you with filling out forms, and walks you through the EA process*

Myth: It takes too long to get the FDA to grant my request

Fact: *Review times are very fast and a "granted" letter is usually sent within one day for both emergency and non-emergency requests*

Myth: Most drug companies don't participate in EA

Fact: *Many drug companies participate in EA and are required to state if they do on their website. Project Facilitate can assist in helping you find a point of contact for a company if needed.*

Compassionate Use

Single Patient IND

Expanded Access

Emergency IND

Pre-Approval Access

Treatment IND

What is Expanded Access?

- Use of an investigational medical product to **treat a patient** with a serious disease or condition who does not have comparable or satisfactory alternative therapies to treat the disease or condition
- Contrast with investigational medical product in a clinical trial where the **primary intent is research**
 - systematic collection of data with the intent to analyze it to learn about the investigational medical product

Access to Treatments

Approved Drugs

- Studied and characterized
- Labeled
- Broadest availability
- Reimbursement is by 3rd party

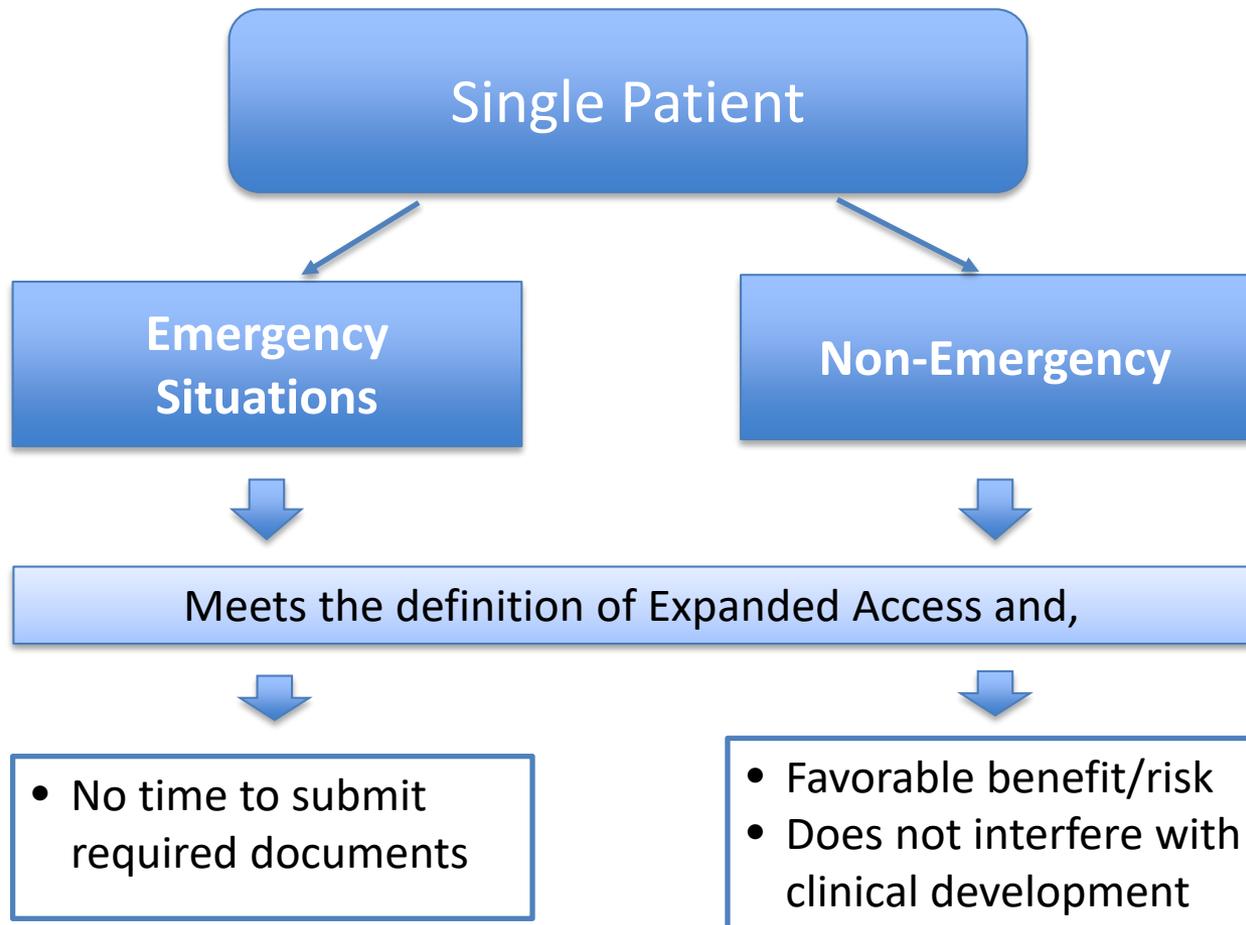
Clinical Trials

- Provide necessary data to determine safety and effectiveness
- Most efficient path to market and broad availability
- <https://clinicaltrials.gov/>

Expanded Access

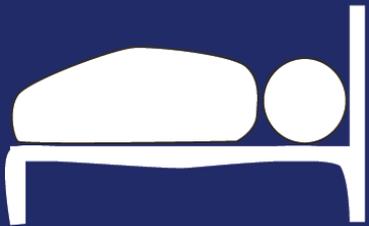
- Represent opportunity when other options are exhausted; Last resort
- Goal is access for treatment
- <https://www.fda.gov/drugs/investigational-new-drug-ind-application/physicians-how-request-single-patient-expanded-access-compassionate-use>

Expanded Access Provisions (FDCA Sec. 561)



Eligibility criteria

Immediate
life-threatening or
serious disease/
condition



Not eligible or
cannot access a
clinical trial



Exhausted all other
treatment options



Physicians Responsibilities



Agrees to oversee the patient's treatment and works with industry (e.g., medical product developer), files paperwork with FDA and IRB, and is responsible for patient care and reporting.

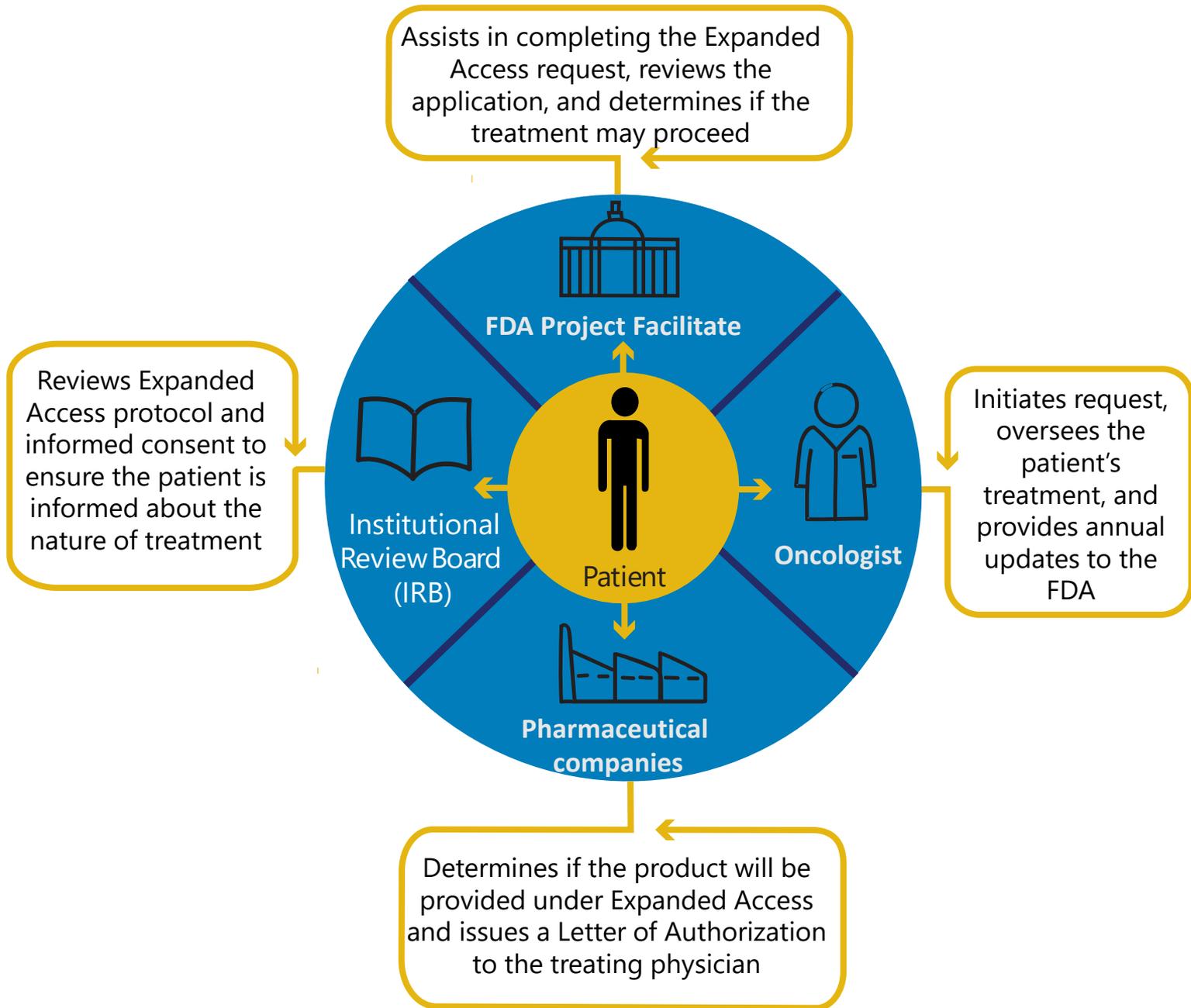
Considerations

- Unknown risks associated with access to investigational products for which there is limited information about safety and effectiveness
 - Some patients may benefit
 - Some patients may experience no effect
 - Some patients may have serious adverse events
- FDA considers:
 - Potential harm to patient
 - Need to exhaust all existing approved therapies
 - Scientific likelihood of an efficacious response



Potential Benefits

- **Access** for oncology patients with serious or life-threatening diseases who have no other alternatives, and are willing to accept greater risk
- **Patient autonomy** over their health care decision
- **Bridge the gap** between the latter stages of product development and approval by making a drug widely available during that period
- **May provide data** to support development

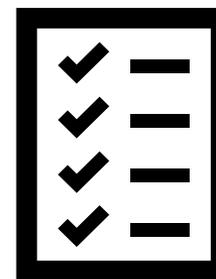


Project Facilitate

- Launched on June 3, 2019
- Call center to assist ***oncology healthcare providers*** in submitting an expanded access request

Project Facilitate Mission

The mission of Project Facilitate is to promote equitable access to investigational products for patients with cancer by providing comprehensive support to oncology healthcare professionals in completing expanded access requests.



Addressing Barriers

Unknowns

- Number of patients requesting Expanded Access
- Reasons for Industry (pharmaceutical companies) denials
- Patient outcomes: benefits, adverse events

Knowns

- Potential unequal access due to lack of information
- Perception of burdensome process/lack of resources

Two-Pronged Approach for Oncology

Patient awareness and information on specific programs

- *Reagan-Udall Expanded Access Navigator* website provides information on sponsors' policies and listings on [ClinicalTrials.gov](https://clinicaltrials.gov)

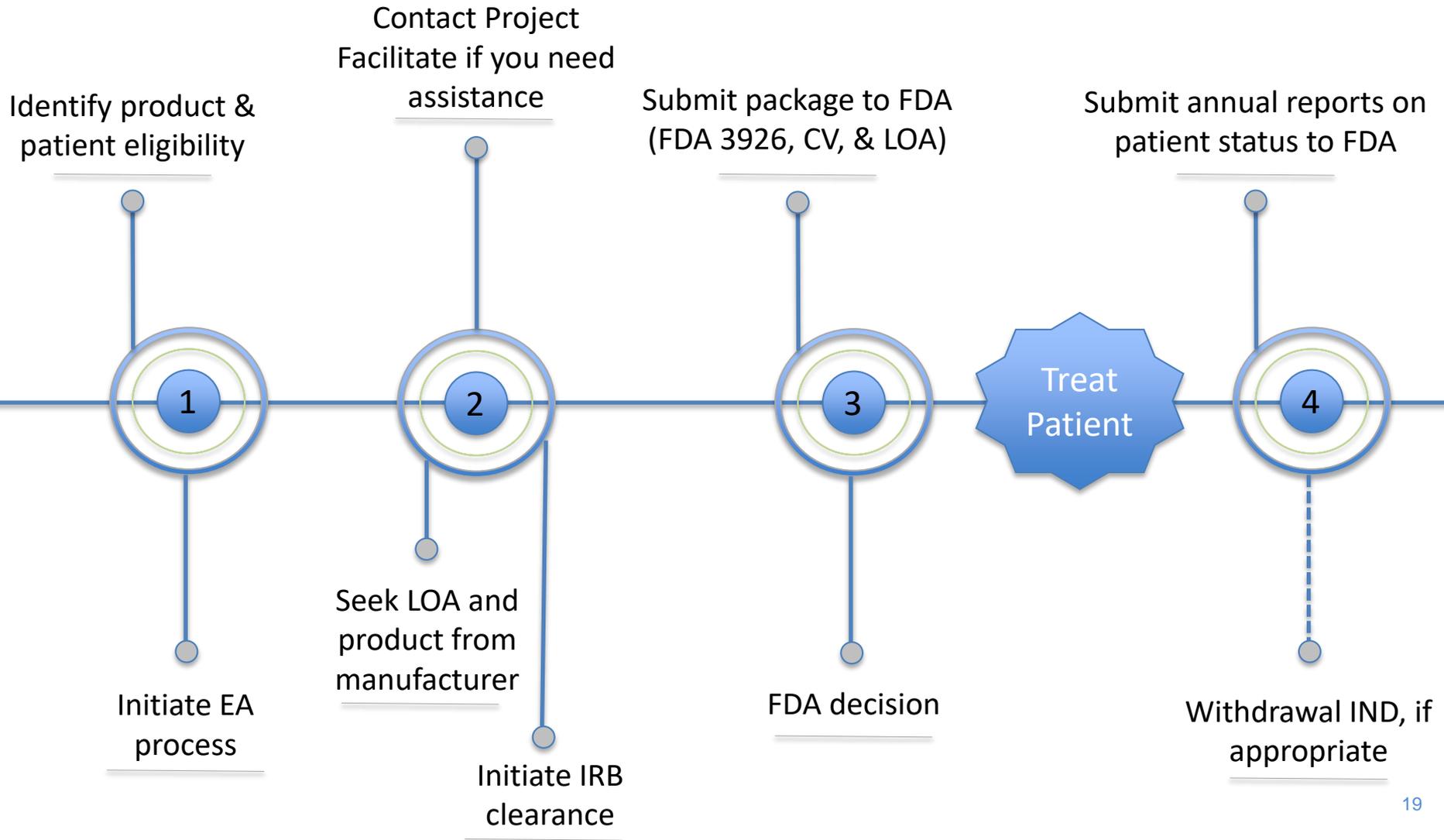
Oncology healthcare provider access

- *Oncology Center of Excellence Project Facilitate* program to provide continuous support to healthcare professionals and their teams throughout the EA process

Benefits of Project Facilitate

- One point of contact for all oncology single patient and emergency requests
- Dedicated clinical staff available during business hours to support requestors via phone or email
- Additional support options to provide:
 - IRB options
 - EA contact for drug/biotech company
 - Assistance filling out Form FDA 3926, if needed
- Efficiency in processing
- Collection of metrics
- Annual report reminders/Follow up

Treating Oncologist Process



Key Points: Industry Agreement

- Locate industry contact
 - Inquire if an expanded access program exists for the product of interest
 - Work with industry contact to get:
 - Letter of Authorization (LOA)
 - Investigational Product
- ❖ If they deny your request to the investigational product, provide the rationale to Project Facilitate

Key Points: FDA Submission

- Letter of Authorization (LOA)
 - Authorization to cross-reference commercial IND
 - Confirmation that the Manufacturer will provide the product
- Form FDA 3926
 - CV of treating Oncologist or statement of qualifications
 - Treatment plan (dose, route, schedule of administration, planned duration, and monitoring plan)
 - *Brief* clinical history of patient
- CV of Treating Oncologist

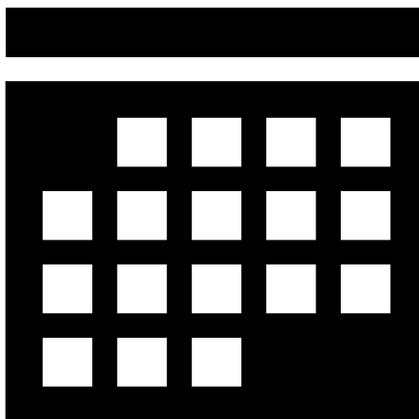
7. Physician's Qualification Statement *(Including medical school attended, year of graduation, medical specialty, state medical license number, current employment, and job title. Alternatively, attach the first few pages of physician's curriculum vitae (CV), provided they contain this information. If attaching the CV electronically, use normal PDF functions for file attachments.)*

*Submit Package to: ONCProjectFacilitate@fda.hhs.gov

When can treatment begin?

For any new access IND, use can begin 30 days after FDA receives the IND (or on earlier notification by FDA) and IRB review has been obtained, except for:

Emergency IND - use can begin when use is authorized by the FDA reviewing official (*often done by phone*).



Key Points: Follow Up

- For individual patient expanded access, the regulations ([21 CFR 312.310\(c\)\(2\)](#)) specify that after treatment the sponsor must provide to FDA a written summary of the results of the expanded access use, including adverse effects
- Provide contact (regulatory, nurse, pharmacist, nurse practitioner/physicians assistant, oncologist, etc.) at initial request for expanded access

Submitting follow-up amendments to the FDA:

- CDER NextGen Portal
- Mail

Reagan-Udall Foundation: Expanded Access Navigator

Created by the Reagan-Udall Foundation
for the FDA, the EA Navigator:

- Provides clear, factual information in an online platform
- Takes users step-by-step through the process of expanded access requests
- Serves as a roadmap for single-patient expanded access requests that inform patients, physicians and companies exploring EA



EXPANDED ACCESS NAVIGATOR

FDA

Expanded Access (EA) may be considered for patients who have exhausted their treatment options and are not eligible for, or able to participate in, a clinical trial.

EA - also known as compassionate use, named-patient use, or single-patient access - provides some patients who have serious or life-threatening diseases or conditions with access to investigational treatments not approved by the U.S. Food and Drug Administration (FDA). The Reagan-Udall Foundation's Expanded Access Navigator provides physicians, patients, and caregivers with guidance on EA and related topics. Scroll down to begin using the Navigator.



- Features portals for providers, patients/caregivers and companies
- Explains role of FDA in expanded access and importance of reporting requirements
- Connects providers, patients and caregivers to investigational therapies
- Supplements Project Facilitate
- Allows companies to demonstrate compliance with laws mandating public expanded access policies
- Promotes greater patient equity

Company Directory

Use this Company Directory to identify investigational drugs, link to clinical trials, and search for expanded access policies, company contact information, and program listings.



ABOUT ▾ GUIDES ▾ COMPANY DIRECTORY ▾ RESOURCES eREQUEST

COVID-19



A B C D E F G H I J K L M N O P Q R S T U V W X Y Z

Company Name

Phone Number & Email

Company Acknowledgement

Abbvie

EA Webpage

Clinicaltrials.gov

AbbviePAA@abbvie.com

2 business days

Expanded Access Listings



Achillion Pharmaceuticals

EA Webpage

Clinicaltrials.gov

(215) 709-3040
globalmedicalaffairs@achillion.com

N/A





New Expanded Access eRequest App Allows Physicians to Submit EA Requests Online

Submitting expanded access requests to FDA just got easier for physicians. The Reagan-Udall Foundation for the FDA, with input from experts at FDA, today launched [Expanded Access eRequest](#) to streamline expanded access for individual patients in non-emergency settings.

“Time is critical when patients have a serious or life-threatening disease or condition and do not have other therapeutic options,” said FDA Principal Deputy Commissioner Amy Abernethy, MD, PhD. “We want to make it easier for physicians to apply for expanded access for their patients and allow these health care providers to focus on the clinical aspects of care.”

The eRequest online app walks physicians and other healthcare providers screen-by-screen through the expanded access process – from determining if expanded access is appropriate for their patient to submitting the request to FDA. eRequest’s home on the Foundation’s Expanded Access Navigator means physicians can identify potential investigational therapies; access sponsor information; complete, sign, and submit FDA Form 3926; upload supporting documentation; and review additional resources all in one place. The app is compatible with multiple devices, so physicians can explore expanded access in real time with their patient and then submit the request when completed.

“The Expanded Access eRequest helps physicians and other prescribers travel easily through a process that can be confusing,” says Susan C. Winckler, RPh, Esq., CEO of the Foundation. “We are pleased to work with FDA to simplify the journey for physicians, and ultimately, for patients.”

Expanded Access Navigator

Expanded Access (EA) may be considered for patients who have exhausted their treatment options and are not eligible for, or able to participate in, a clinical trial.



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[RESOURCES](#)

[eREQUEST](#)

[COVID-19](#)



Expanded Access - also known as compassionate use, named-patient use, or single-patient access - provides some patients who have serious or life-threatening diseases or conditions with access to investigational treatments approved by the U.S. Food and Drug Administration (FDA). The Reagan-Udall Foundation's Expanded Access Navigator provides physicians, patients, and caregivers with guidance on EA and related topics. Scroll down to begin using the Navigator.



Select the step-by-step guide best matching your needs.



Expanded Access eRequest

Physicians: Electronically request **non-emergency** use of medical products for your patients

[Get Started](#)

Not a Physician? [View Information for Patients and Caregivers](#)



Expanded Access Resources

- Patients and Non-Oncology Healthcare Professionals
 - Reagan Udall Foundation
 - Website: <https://reaganudall.org/>
 - FDA Division of Drug Information (DDI)
 - Website: <https://www.fda.gov/news-events/expanded-access/fdas-expanded-access-contact-information>
 - (855) 543-DRUG or druginfo@fda.hhs.gov
- Oncology Healthcare Professionals
 - Project Facilitate
 - Website: <https://www.fda.gov/about-fda/oncology-center-excellence/project-facilitate>
 - (240) 402-0004 or ONCProjectFacilitate@fda.hhs.gov
 - Reagan Udall Foundation
 - EA Navigator: <https://navigator.reaganudall.org/expanded-access-navigator>

Phases of Project Facilitate

PHASE 1



Call Center (May 31, 2019)

- Provide resources for the oncologist and healthcare team
- Triage CDER and CBER applications to each respective oncologic division or center for clinical review
- Divisions also received requests directly from the public
- Call center staffed by Regulatory Project Management volunteers from oncology divisions

PHASE 2

Call Center (September 1, 2019)

- Increased designated full-time staff, less reliant on Regulatory Project Management volunteers
- Outreach to clinics, hospitals, conferences and organizations
- Continue to triage applications to oncologic divisions or CBER for clinical review
- Developed an internal request/application tracking database



PHASE 3



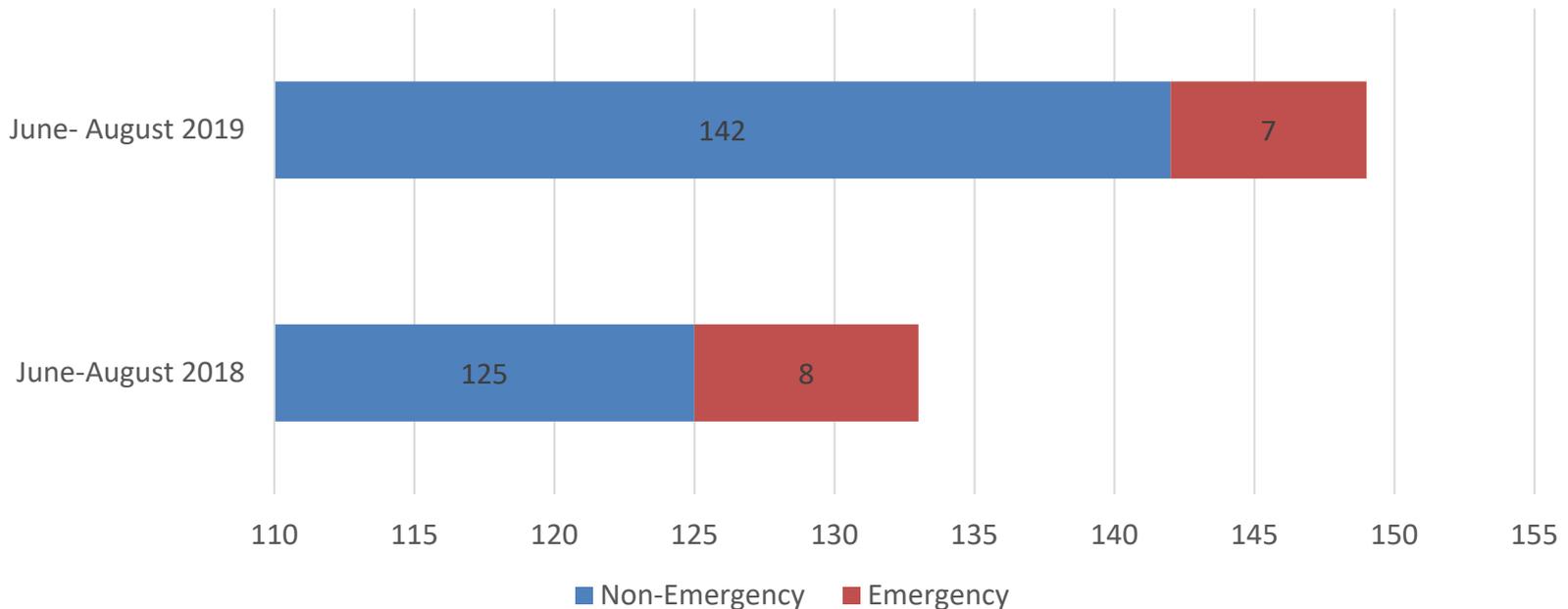
Call Center and Application Review

- April 6, 2020 PF staffed full-time by clinical staff who cover the call center and performed review and management of all CDER EA requests that came directly to PF. The divisions continued to review requests submitted directly to the division
- June 1, 2020 PF began performing review and management of all CDER EA oncology requests.

Project Facilitate 2019 Prelim-Data

- Phase I: May 31, 2019 – August 31, 2019
- Total Inbound Calls: 172
- E-Mails: 1,007

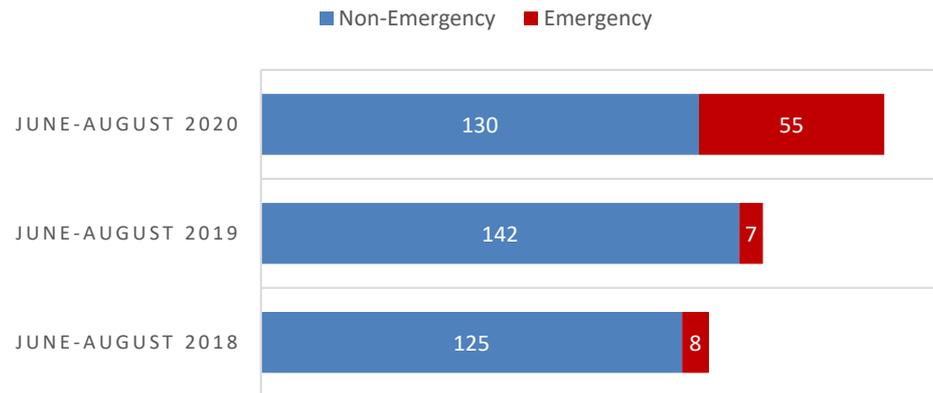
OHOP Single Patient Expanded Access Requests



Project Facilitate 2020 Comparison

- Data Comparison:
 - May 31, 2018 – August 31, 2018
 - Applications: 133
 - May 31, 2019 – August 31, 2019
 - Applications: 149
 - Total inbound calls: 172
 - Emails: 1,007
 - May 31, 2020 – August 31, 2020
 - Applications: 185
 - Total inbound calls: 157
 - Emails: 1,229

OHOP/OOD SINGLE PATIENT EXPANDED ACCESS REQUESTS



Project Facilitate Phase 3

- Review and management of all CDER oncology EA requests



Emails: 3454

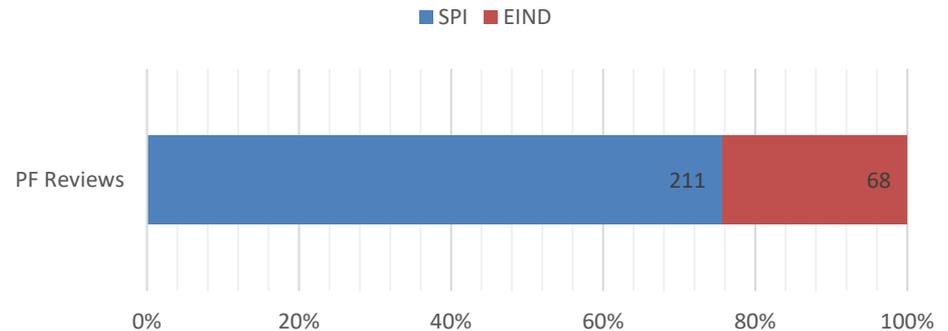


Received Phone Calls: 247



3 Full Time Clinical Staff

Project Facilitate Reviewed Applications
June 1, 2020 - October 31, 2020

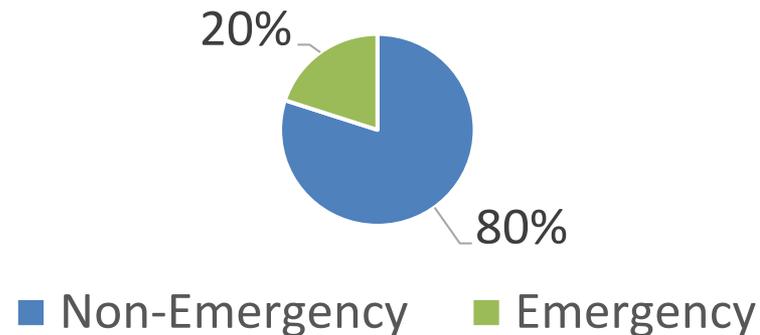


Project Facilitate Review

- Published at ASCO 2020
- 573 phone calls received May 31, 2019 to March 31, 2020
- 1,336 INDs from May 31, 2018 – March 31, 2020 were analyzed

<p>N=1,336 Non-emergency: 1,065 Emergency: 271</p>
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Figure 1: Single Patient EA Request Summary



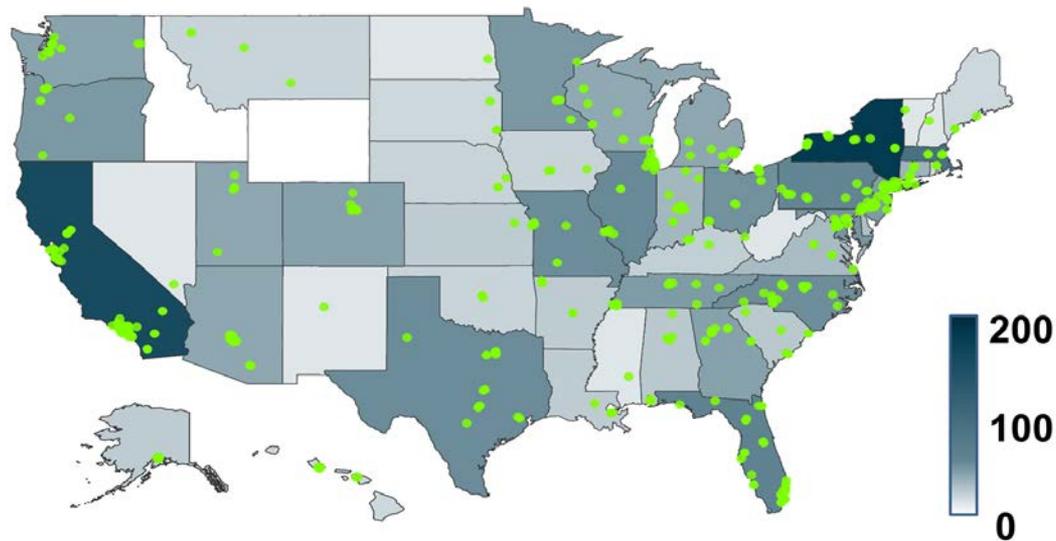
Project Facilitate Review

- Processing time date range
 - Pre-PF: May 31, 2018 to March 31, 2019
 - Post-PF: May 31, 2019 to March 31, 2020
 - Total: May 31, 2018 to March 31, 2020
- FDA review time improved by a mean of 0.9 days
 - 111 of 1,336 (8%) took >5 business days for FDA determination

	Pre-Project Facilitate N=380	Project Facilitate N=814	Total N=1,336
Non-Emergency EA Mean (SD) Business Days	2.5 (3.7)	1.6 (2.2)	1.9 (2.8)
Emergency EA Mean (SD) Business Days	0.7 (1)	0.6 (1.1)	0.8 (1.2)
Combined EA Mean (SD) Business Days	2.1 (3.3)	1.4 (2.1)	1.7 (2.6)

Project Facilitate Review

- Volume of EA requests increased by 434 (36.4%) during the PF timeframe compared to pre-PF
- Highest number of requests originating from NY, CA, TX, MA, and FL



Conclusion

- **Expanded Access** can provide patients who have exhausted other treatment modalities the use of an investigational product.
- **Manufacturer** must be willing to provide the product.
- **Project Facilitate** assists oncology healthcare providers in processing the FDA expanded access request and providing personalized support
- **Reagan-Udall Foundation** is a useful resource for both oncology healthcare providers and for patients/caregivers.
- **eRequest** is a new feature located on the Reagan-Udall Foundation website where requests can be submitted online through an easy-to-use interface
- **Project Facilitate** improves response and review times for oncology expanded access applications compared to prior years

References

Lemery, S., Mailankody, S., Kazandjian, D., Demiette Smit, M., Blumenthal, G., Kim, T., Keegan, P., McKee, A., Pazdur, R. (2016, June). *Food and Drug Administration analysis of 1332 single patient and emergency use expanded access (compassionate use) requests for patients with cancer over a duration of three years (2012-2014)*. Abstract accepted and poster presented as American Society of Clinical Oncology Annual Meeting, Chicago, IL.

Project facilitate: A review of the FDA oncology center of excellence expanded access pilot program. Natasha L Kormanik, Mitchell Chan, Jessica Boehmer, Tamy Kim, Gideon Michael Blumenthal, and Richard Pazdur
Journal of Clinical Oncology 2020 38:15_suppl, 7023-7023

Reagan-Udall Foundation (n.d.). *Expanded Access Navigator*. Retrieved from <https://reaganudall.org/>

Useful Resources

- FDA Project Facilitate Website: <https://www.fda.gov/about-fda/oncology-center-excellence/project-facilitate>
- FDA Expanded Access Site: <https://www.fda.gov/news-events/public-health-focus/expanded-access>
- Reagan-Udall Foundation EA navigator: <https://navigator.reaganudall.org/expanded-access-navigator>
- eRequest: <https://erequest.navigators.reaganudall.org>
- Form 3926: <https://www.fda.gov/media/98616/download>
- Instructions for 3926: <https://www.fda.gov/media/98627/download>
- FDA Drug Info Rounds Expanded Access Video Series: <https://www.fda.gov/drugs/information-healthcare-professionals-drugs/fda-drug-info-rounds-expanded-access-video-series>

**FDA U.S. FOOD & DRUG
ADMINISTRATION**

ONCOLOGY CENTER OF EXCELLENCE

Project Facilitate

Assisting healthcare providers with requests for access to investigational oncology products

**DO YOU NEED HELP SUBMITTING A SINGLE PATIENT IND EXPANDED ACCESS (EA) REQUEST
(ALSO KNOWN AS COMPASSIONATE USE) FOR A PATIENT WITH CANCER?**

...FDA's Oncology Center of Excellence (OCE) can help:

- Locate IRB resources
- Find an EA contact for a drug/biotech company
- Complete Form FDA 3926

8:00 AM - 4:30 PM Eastern Time (M-F)

Phone: (240) 402-0004

Email: OncProjectFacilitate@fda.hhs.gov

www.fda.gov/oce



Patients: Talk to your healthcare provider to discuss whether expanded access is an appropriate option.

After Hours Emergency Requests: Contact FDA's emergency call center at (866) 300-4374

